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Online survey study to explore patient and caregiver preferences for different spinal muscular atrophy treatments

Submission date
08/01/2021

Recruitment status
No longer recruiting

Registration date
15/01/2021

Overall study status
Completed

Last Edited
18/07/2022

Condition category
Musculoskeletal Diseases

[ ] Prospectively registered
[ ] Protocol
[ ] Statistical analysis plan
[X] Results
[ ] Individual participant data

Plain English summary of protocol

Background and study aims
Spinal muscular atrophy (SMA) is a genetic condition that makes the muscles weaker and causes problems with movement. It's a serious condition that gets worse over time, but there are treatments to help manage the symptoms.
This is a non-treatment, online survey study designed to explore patient and caregiver preferences for different treatment attributes for SMA Type II and non-ambulatory Type III. The study will be conducted in five European countries: the Netherlands, Finland, Ireland, Portugal and Belgium.

Who can participate?
Parents/caregivers of patients aged <18 years and adult patients living with SMA type II and non-ambulatory type III.

What does the study involve?
Patient/caregiver participants will be recruited through patient associations in each of the five target countries. Potential participants will be sent a link to the survey via email. Participants will first be asked some screening questions to determine their eligibility. If eligible, participants will be given further information about the study and asked to give their informed consent online before proceeding to the survey.
In the survey, participants will be asked a series of clinical questions about their or their child's SMA diagnosis and treatment alongside demographic and background questions in relation to the SMA patient (either themselves or their child) and, if applicable, the caregiver themselves (caregiver participants only). This will be followed by a presentation of treatment attributes and a DCE survey to assess their preferences for SMA treatments.
The survey will last approximately 20-30 minutes.

What are the possible benefits and risks of participating?
No treatment will be provided in this study; therefore, there are no known physical risks. However, during or following participation in the study, patient and caregiver participants may be reminded and become more aware of their or their child's condition. There will be no direct

benefit to the participant.
The hope is that study results will lead to a robust assessment of patient and caregiver preferences for SMA treatment attributes.

Where is the study run from?
This is an online study in the Netherlands, Finland, Ireland, Portugal and Belgium.

When is the study starting and how long is it expected to run for?
May 2020 to APril 2021.

Who is funding the study?
F. Hoffmann-La Roche Ltd (USA)

Who is the main contact?
global-roche-genentech-trials@gene.com, reference Study ID ALC 1079

Contact information

Type(s)
Public

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Additional identifiers

Protocol serial number
ALC 1079

Study information

Scientific Title
Patient and carer preference weights in spinal muscular atrophy: a discrete choice experiment (DCE) survey in five European countries

Study objectives
To explore and quantify the value of, or strength of preference for Risdiplam’s treatment characteristics compared to the treatment characteristics of Spinraza and Zolgensma among parents/caregivers of patients aged <18 and adult patients living with SMA type II and non-ambulatory type III.

Ethics approval required

Old ethics approval format

Ethics approval(s)
Date of IRB exemption status determination: 19/08/2020. Western Institutional Review Board. This is a non-interventional study that does not utilize drugs, and will not take place at any centres. It is strictly an online survey.

Primary study design
Observational

Study design
Non-interventional online survey study

Study type(s)
Other

Health condition(s) or problem(s) studied
SMA Type II and non-ambulatory Type III

Interventions
Approximately 70 survey participants will be recruited through participant associations within the five target countries. Potential participants will be contacted by e-mail with information about the study. If interested, participants will complete a brief screener questionnaire to check whether they meet the inclusion criteria.

Eligible participants will be provided with an information sheet about the study describing the study content, study procedures and their rights as participants and asked to complete a consent form in order to take part in the survey should they wish to do so. Participants will then proceed to the main survey. The survey consists of two parts: the first part of the survey will collect clinical and demographic details of the patient and, if applicable, demographics of the caregiver (caregiver participants only); the second part of the survey will be a DCE consisting of a series of choices between hypothetical treatments varying in terms of treatment attributes, including treatment benefits, side effects, mode of administration, and level of evidence available for different treatment options.

The survey is expected to take approximately 20-30 minutes to complete. Participants will not receive a study remuneration for their participation in the study.

Intervention Type
Other

Primary outcome(s)
Relative importance of treatment characteristics measured using a DCE survey at a single time point

Key secondary outcome(s)
There are no secondary outcome measures

Completion date
30/04/2021

Eligibility

Key inclusion criteria
1. A patient self-reported or caregiver proxy-reported diagnosis of SMA Type II: age of onset between 7 and 18 months OR SMA Type III: age of onset 18+ months and 17 years
2. Non-ambulatory, defined as unable to walk more than ten steps without a form of assistance
3. Adult patients aged 18+ OR caregivers of patients aged <18 years
4. Residents of the Netherlands, Finland, Ireland, Portugal or Belgium
5. Willing and able to give their informed consent

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Mixed

Sex
All

Key exclusion criteria
Does not meet inclusion criteria

Date of first enrolment
01/10/2020

Date of final enrolment
31/03/2021

Locations

Countries of recruitment
Belgium

Finland

Ireland

Netherlands

Portugal

United States of America

Study participating centre

Online survey administered by Genentech, Inc.
1 DNA Way
San Francisco
United States of America
94080

Sponsor information

Organisation
Roche (United States)

ROR
https://ror.org/011qkaj49

Funder(s)

Funder type
Industry

Funder Name
Genentech

Alternative Name(s)
Genentech, Inc., Genentech USA, Inc., Genentech USA

Funding Body Type
Government organisation

Funding Body Subtype
For-profit companies (industry)

Location
United States of America

Results and Publications

Individual participant data (IPD) sharing plan
The datasets generated during and/or analysed during the current study are not expected to be made available as they are not required to be made available.

IPD sharing plan summary
Not expected to be made available

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article		13/12/2021	18/07/2022	Yes	No